

Supplementary table 2: miHTT expression in off-target organs a) NHP and b) rat

a)

| Dose, gc/brain | miHTT expression in off-target organs in NHP (gc/ μ g of RNA) | | | | | | |
|--------------------|---|---------------------|-----------------------------|--|--|--------------|--|
| | Adrenal gland | Cervical lymph node | Heart | Kidney | Liver | Spleen | Ovaries |
| 2x10 ¹² | n=6 <LLOQ | n=6 <LLOQ | n=6 <LLOQ | n=5 4.04x10 ³ (9.98x10 ²) | n=6 <LLOQ | n=6 <LLOQ | Not analyzed |
| 7x10 ¹² | n=2 4.73x10 ³ (2.48x10 ³) | n=6 <LLOQ | n=1 5.11x10 ³ | n=1 3.34x10 ³ | n=2 4.79x10 ³ (1.51x10 ³) | n=6 <LLOQ | Not analyzed |
| 2x10 ¹³ | n=3 6.12x10 ³ (3.54x10 ³) | n=6 <LLOQ | n=1 4.22x10 ³ | n=6 <LLOQ | n=4 5.57x10 ³ (1.38x10 ³) | n=6 <LLOQ | n=3 2.35x10 ⁴ (2.22x10 ⁴) |

Data are mean (standard deviation). N=6 for all tissues except ovaries (N=3). Where lower, n represents positively determined concentration of vector DNA. LLOQ was 25 copies/reaction. gc, genome copies; LLOQ, lower limit of quantification; miHTT, micro RNA targeting *HTT*; NHP, non-human primates.

b)

| Time, months post dose | miHTT expression in off-target organs in rats (gc/ μ g of RNA) | | | | | |
|------------------------|--|-----------------------------|---------------|--|--|--|
| | Adrenal gland | Cervical lymph node | Liver | Lung | Spinal cord | Spleen |
| 1 | n=6 1.80x10 ⁴ (1.14x10 ⁴) | n=1 2.86x10 ³ | n=10 <LLOQ | n=10 <LLOQ | n=10 <LLOQ | n=10 <LLOQ |
| 6 | n=7 2.63x10 ⁴ (3.30x10 ⁴) | n=10 <LLOQ | n=10 <LLOQ | n=2 3.98x10 ³ (6.15x10 ²) | n=5 4.81x10 ³ (1.43x10 ³) | n=2 5.54x10 ³ (3.93x10 ³) |

Data are mean (standard deviation). N=10 for all tissues. Where lower, n represents positively determined concentration of vector DNA. LLOQ was 2.5x10³ copies/ μ g of RNA. gc, genome copies; LLOQ, lower limit of quantification; miHTT, micro RNA targeting *HTT*.